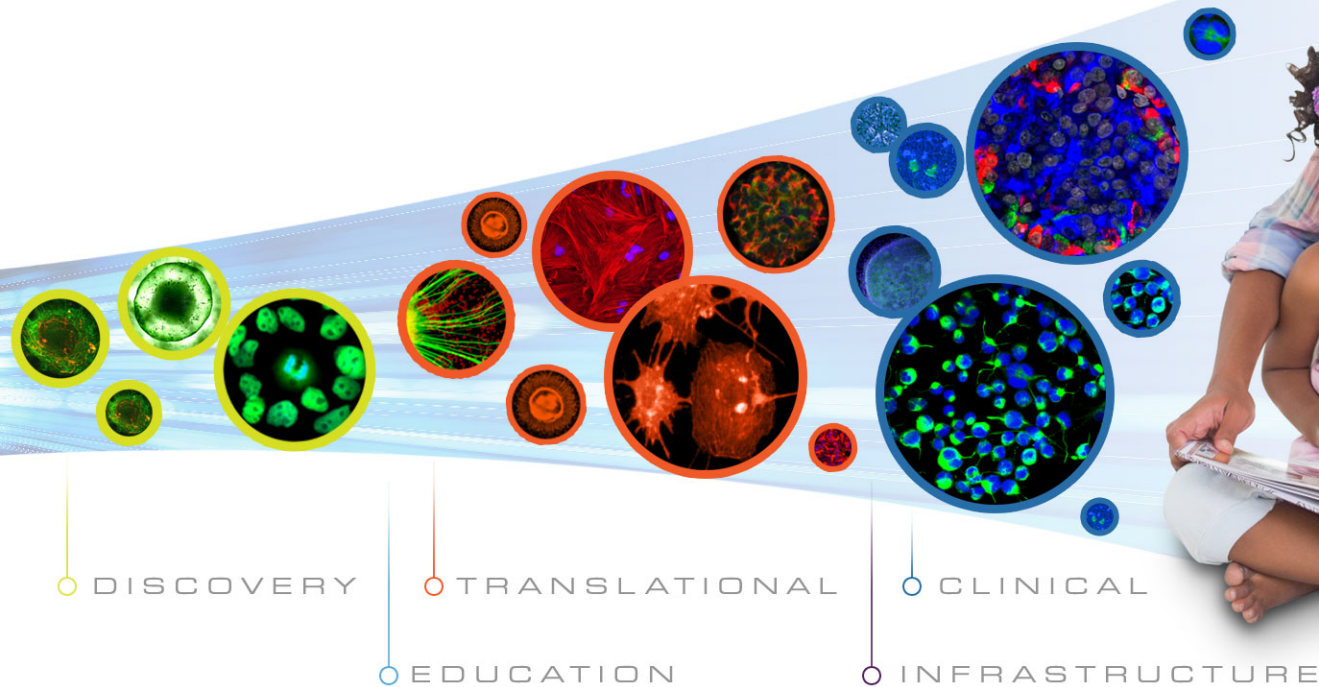


Dec. 14, 2017

CIRM

CALIFORNIA'S STEM CELL AGENCY



2017 4th Quarter Update

Presentation to the Board of Directors

Maria T. Millan, M.D.
President and Chief Executive Officer
California Institute for Regenerative Medicine

CIRM's Mission

Accelerate stem cell
treatments to patients with
unmet medical needs

Agenda

- 2017 Highlights & Update on Strategic Plan
- Budget
- Proposal and Requested Action
 - CLIN award CAP Reduction
 - 2018 Budget approval

2017 Highlights

Cell therapy is coming of age

- Marketing approvals for Novartis and Kite/Gilead's CAR-T products
- FDA Regulatory Reform under the 21st Century Cures Act
- NIH – CIRM Partnership Opportunities

2017 Highlights

RMAT Expedited Regulatory Pathway

- 21st Century Cures Act of Dec 2016 created the expedited FDA pathway RMAT launched in Q1 2017
 - Stem cell, gene therapy, regenerative medicine, tissue engineered
 - Nimble process
 - Combines breakthrough designation and accelerated pathway
- CIRM grantees among the first in the U.S. to obtain RMAT designation
 - Humacyte- engineered vascular graft for kidney failure
 - jCyte- cell therapy for blinding eye disease
 - Asterias- cell therapy for spinal cord injury

2017 Highlights

CIRM-NIH Partnership

- NIH Cures Act-funded Regenerative Medicine Innovation Program
 - Dec. 6-7 NIH-FDA Regenerative Medicine Innovation Workshop
 - NIH \$30M/5yrs for adult stem cells
- CIRM-NIH joint site visit for NHLBI Sickle Cell Cures Initiative
 - \$1.7M 2-yr. award to COH for GMP-compatible gene editing
- CIRM involvement in NIDCR multi-center award
 - Harmonization of project objectives so successful researchers would be eligible and competitive for CIRM's TRAN program

2017 Update



Infrastructure



Education



Discovery



Translation



Clinical

CIRM Funding

2017 Update

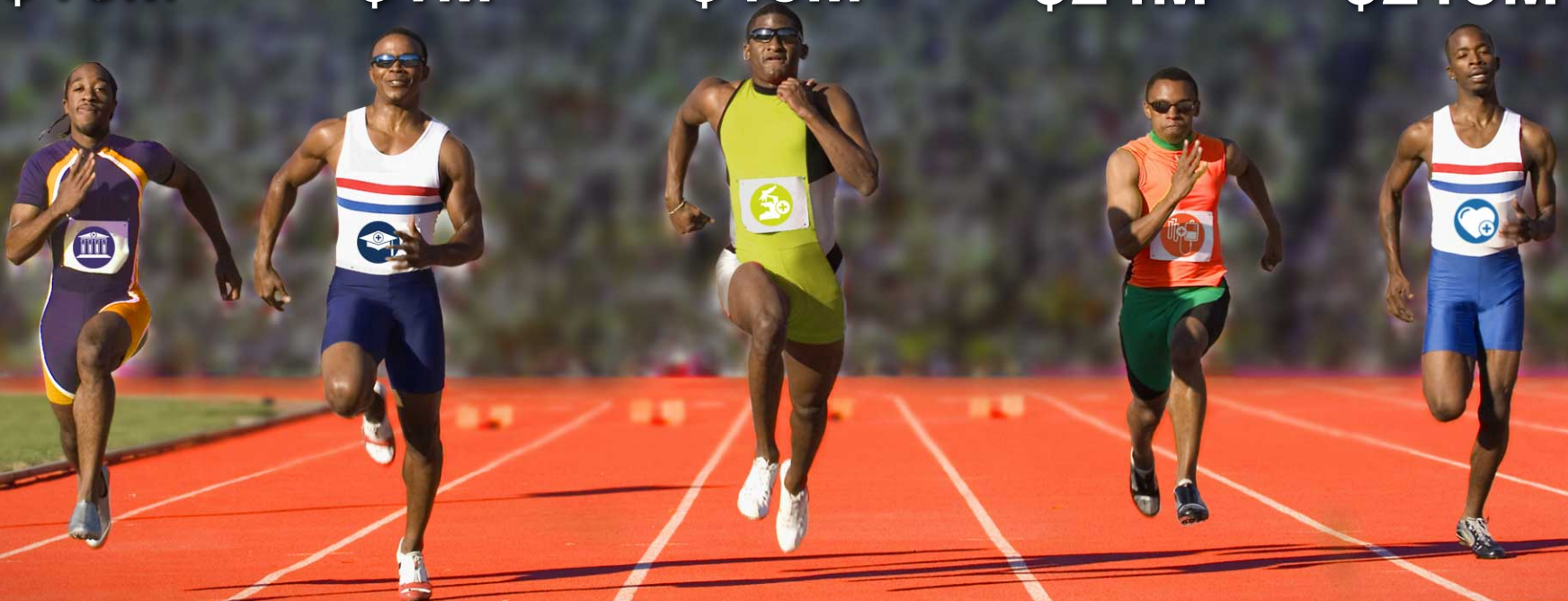
Infrastructure
\$16M

Education
\$1M

Discovery
\$45M

Translation
\$24M

Clinical
\$213M



5 Pillars of investment to Accelerate Science to Cures

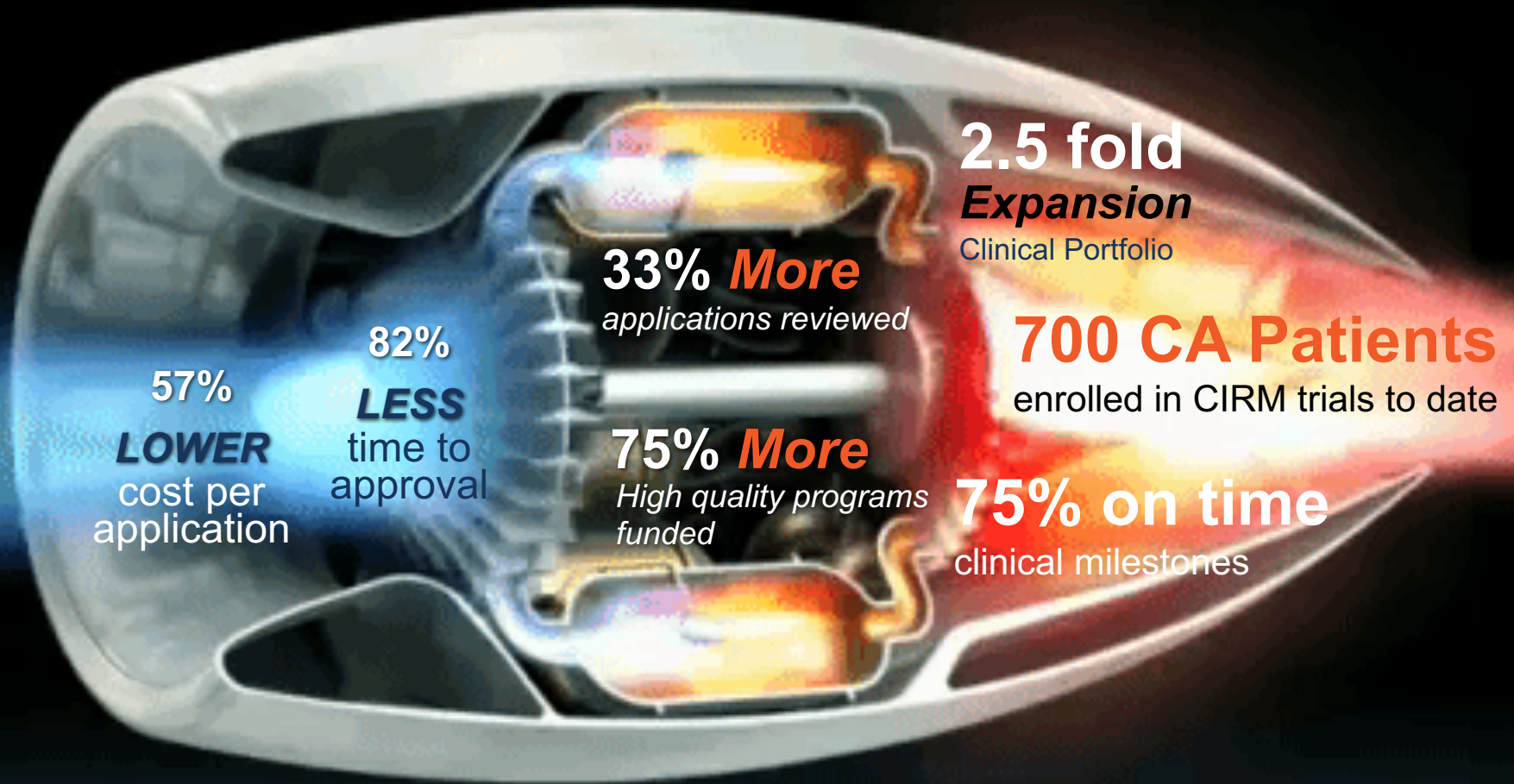
Every Moment Counts | Don't Stop Now

8

CIRM
CALIFORNIA / STEM CELL AGENCY

Acceleration by Design

2017 Performance



2017 Update on Strategic Plan:

Clear and on Target

Ahead

24 of 50 New
Candidates

DISCOVER

Exceed

2-fold
increase in
Progression

ADVANCE

Lead

Expedited
Regulatory
Path

25% RMATs

REFINE

Gaining

3 INDs in
<18 Months

ACCELERATE

Ahead

26 of 50
New trials

VALIDATE

Increase

6 Industry
Investments
in 2017

PARTNER

Wheels up
and powered to
Break Barriers

\$306.7M

2017

\$125M

2016

\$41.3M

2015

Private Industry Investment

Leveraging CIRM Investment to Date

\$911M*^a

Co-funding
by applicant

\$473M^a

Resulting
Partnership Funding

>\$395M^a

Additional funds due
to CIRM award

* Including \$50M from non CA

^a Approximate amounts

Agenda Item #5

CIRM Budget Update

January 1st, 2018



Research

+



Administration

Research Budget

2017 Update

- 5-year Strategic Plan launched in 2016 with a \$890M Budget
 - Success of the Clinical Program has led to faster than expected expenditure
 - Estimate last awards by Q4 2019 vs. original Q2 2020 projection
- Strategic Plan projected \$440M for 50 clinical trials
 - \$300M already expended on 26 trials + 9 CLIN1s
 - Average CLIN trial award increased from \$10.9 in 2015/2016 to \$12.1M in 2017
- Administrative & Research budgets to be fully expended mid 2020
 - Final Research awards will be made in 2019; Administrative Budget available beyond 2020

2017 CIRM Research Awards

Program	2017 Allocation	Est 2017 Total *
Education	\$1M	\$1M
Discovery	\$52M	\$46M
Translation	\$45M	\$24M
Clinical	Up to \$215M	\$213M
Infrastructure	\$16M	\$16M
Total	\$329M	\$300M

* Est Remaining Approvals: 11 DISC2s for \$21.1M

2017 Year End Budget Update

As of January 1, 2017

- Committed / Uncommitted Balance \$2.22B / \$528M

2017 Estimated Activity

- 88 Awards Added \$299.9M
- Recovery from unexpended funds \$41M

As of December 31, 2017

- Committed / Uncommitted \$2.48B / \$269M

Future Recovery (2018-2019) \$66M

Available Big Bucket research funds (total) \$335M

Budget Planning

Operating Principles

- Execute on 5-year Strategic Plan approved by the ICOC on December 2015
- Critical personnel level required to execute on the Strategic Plan while maintaining operational excellence
- Essential to preserve CIRM's value proposition to increase the probability of and the speed by which stem cell treatments can reach patients

Research Budget Background:

- \$335M left in Research with last awards in Q4 2019
- With the current CLIN award budgets and a goal of 50 new clinical trials, we would only have enough research funds for CLIN programs (no additional DISC or TRAN awards)
- Reduction in CLIN award caps would generate ~\$68M in savings to fund additional DISC and TRAN programs

CLIN Award Caps:

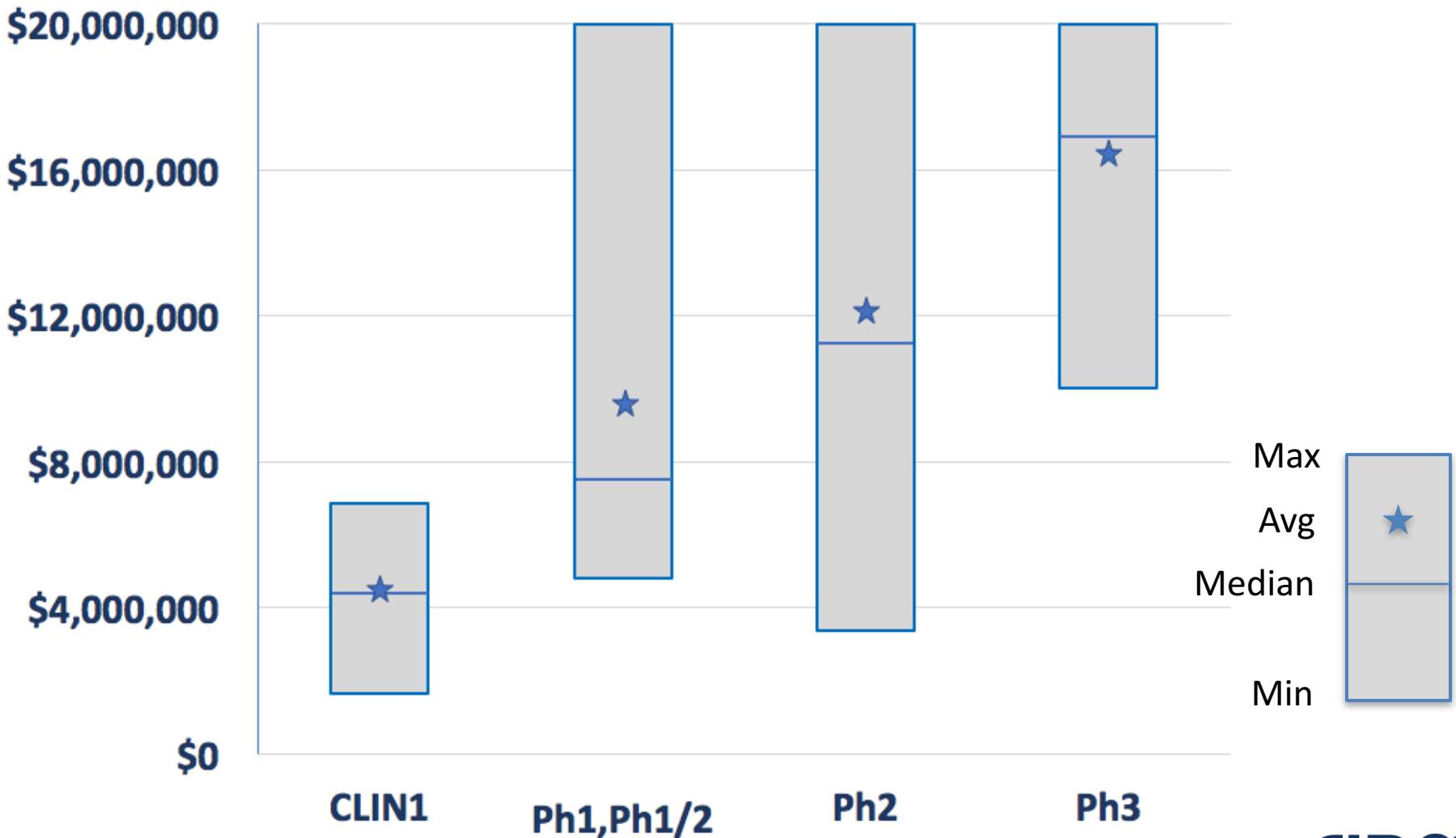
Current Status and Proposed Caps

CLIN Program	CLIN1	Phase 1 Phase 1/2	Phase 2	Phase 3
Current CLIN Award Caps*	\$20M	\$20M	\$20M	\$20M
2017 Average Award \$	\$4.9M	\$10.3M	\$15.3M	\$16.7M
Proposed Award Cap Non-Profit For-Profit	\$6M \$4M	\$12M \$8M	\$15M \$15M	\$10M \$10M

* \$20M CLIN Award caps are a holdover from Disease Team Awards which funded a combination of activities currently funded separately under TRAN, CLIN1 & CLIN2 programs.





CLIN Award Sizes (2015-2017)

Averages and Medians



Proposed Research Budget Allocation

with CLIN Award Cap Reduction

		2018	2019
CLIN		\$130M (12 trials + 4 Candidates)	\$130M (12 trials + 4 Candidates)
TRAN		\$30M (6 Candidates)	\$25M (5 Candidates)
DISC		\$10M (8 Projects)	\$9.25M (7 Projects)
EDUC		\$750K	\$0

- Can achieve 50 new clinical trials & 40 new candidates
- DISC and EDU with decreased offerings

CIRM's Research Budget Scenarios:

- Consistent with 5-year strategic goals
- Preserves the full complement of research programs
- Preserves CIRM's "acceleration by design" operations
- Administration Budget* can support the personnel required for this Research Budget Plan.

**Administration Budget scenario presented to the Joint Research and Transition subcommittees in November. Formal Budget request for 18/19 will be brought to the ICOC in March 2018*

Requested Action

The CIRM team requests that the ICOC approve the proposed reduction in the maximum funding level for CLIN awards according to the below:

CLIN Program	CLIN1	Phase 1 Phase 1/2	Phase 2	Phase 3
Non-Profit	\$6M	\$12M	\$15M	\$10M
For-Profit	\$4M	\$8M	\$15M	\$10M

Requested Action

The CIRM team requests that the ICOC approve the 2018 proposed research budget allocation of:

\$130M for CLIN

\$30M for TRAN

\$10M for DISC

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unmet medical needs